

Phase 1 Safety Assessment of CPCB-RPE1, hESC-derived RPE Cell Coated Parylene Membrane Implants, in Patients with Advanced Dry Age Related Macular Degeneration

Grant Award Details

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Grant Type: Disease Team Therapy Development III

Grant Number: DR3-07438

Project Objective: The project is developing a therapy to replace RPE cells in patients with a form of AMD known as geographic atrophy. The RPE cells are delivered on a synthetic membrane, just as normal RPE cells in the eye are arranged as a thin layer of cells on a substrate known as the Bruch's membrane. They have shown that their approach of delivering the RPE as a sheet into diseased animal eyes has a much better chance of restoring retinal function than if these cells were injected as a suspension. In this current grant they are seeking to translate this preclinical work and to test this product in a Phase I clinical trial in patients with geographic atrophy.

Investigator:

Name: Mark Humayun
Institution: University of Southern California
Type: PI

Name: David Hinton
Institution: University of Southern California
Type: Co-PI

Disease Focus: Age-related macular degeneration, Vision Loss

Human Stem Cell Use: Embryonic Stem Cell

Award Value: \$17,128,661

Status: Active

Progress Reports

Reporting Period: Year 1

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Reporting Period: Year 2

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Grant Application Details

Application Title: Phase 1 Safety Assessment of CPCB-RPE1, hESC-derived RPE Cell Coated Parylene Membrane Implants, in Patients with Advanced Dry Age Related Macular Degeneration

Public Abstract: It is estimated that by 2020, over 450,000 Californians will suffer from vision loss or blindness due to the age-related macular degeneration (AMD), the most common cause of retinal degeneration in the elderly. AMD is a progressive ocular disease of the part of the retina, called the macula, which enables people to read, visualize faces, and drive. The disease initially causes distortion in central vision, and eventually leads to legal blindness. A layer of cells at the back of the eye called the retinal pigment epithelium (RPE), provides support, protection, and nutrition to the light sensitive cells of the retina; the photoreceptors. The dysfunction and/or loss of these RPE cells is believed to play a critical role in the subsequent death of photoreceptors and resulting loss of vision in AMD. Hence, if RPE cells can be restored, it may be possible to prevent or delay progressive vision loss in patients with AMD.

We are developing a therapy to replace RPE cells in patients with a form of AMD known as geographic atrophy. The RPE cells are delivered on a synthetic membrane, just as normal RPE cells in the eye are arranged as a thin layer of cells on a substrate known as the Bruch's membrane. We have made excellent progress in the current grant period funded by CIRM and are on track to file to an investigational new drug application to the FDA to start the phase 1 human study. We have also shown that our approach of delivering the RPE as a sheet into diseased animal eyes has a much better chance of restoring retinal function than if these cells were injected as a suspension. In this current grant from CIRM, we are seeking to translate this preclinical work and to test this product in a Phase I clinical trial in patients with geographic atrophy.

Statement of Benefit to California:

Age-related macular degeneration (AMD) is the leading cause of vision loss and blindness among the elderly. It is estimated that over 1.75 million individuals in the US suffer from advanced AMD, and that this number will grow to nearly 3 million by 2020. Based on the demographics of California and the incidence rates of AMD among various age groups, it is anticipated that more than 400,000 Californians will develop advanced forms of AMD over the next 15 years. Although VEGF inhibitors such as Lucentis and Eylea provide therapeutic options for the wet form of AMD, no approved therapies currently exist for the advanced form of dry AMD, geographic atrophy, which is estimated to affect more than 100,000 Californians today, with another 160,000 new cases in California expected in the next 15 years. Studies have shown that the devastating consequences of AMD include the progressive loss of independence and productivity, and increased risks of falls, fractures, and depression among patients, resulting in substantial quality of life and economic impacts to many Californians. One health economics analysis estimated the total GDP from dry AMD in the United States to be more than \$24 billion annually. Given that California accounts for more than 12% of US GDP, this analysis can be extrapolated to estimate that total GDP loss due to dry AMD in California is likely nearly \$3 billion annually.

In the proposed project, phase I clinical testing of an implant composed of an ultrathin membrane coated with human embryonic stem cell-derived retinal pigment epithelial cells (RPE), one of the key cell types known to primarily degenerate or die in AMD, will begin in patients with advanced AMD. This project has been developed in California and clinical testing will begin in California. This project will benefit the state of California by providing treatment options for the hundreds of thousands of Californians with dry AMD, providing support for the further development of a therapy originally developed with California, and creating jobs in California by enabling the formation a new California-based start-up to further develop this product.

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